Curing Sickle cell Disease with CRISPR-Cas9 genome editing

Grant Award Details

Curing Sickle cell Disease with CRISPR-Cas9 genome editing

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-11497

Project Objective: Obtain an IND for an early phase clinical trial

Investigator:

Name:	Mark Walters
Institution:	University of California, San Francisco
Type:	PI

Disease Focus: Blood Disorders, Sickle Cell Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $2,242,805

Status: Pre-Active

Grant Application Details

Application Title: Curing Sickle cell Disease with CRISPR-Cas9 genome editing

Public Abstract:

Therapeutic Candidate or Device

Blood stem cells collected from individuals with sickle cell disease will have the sickle gene corrected and then given back to the same individual.

Indication

Sickle cell disease is a hereditary blood disorder associated with pain and other serious medical complications including a shortened life-span

Therapeutic Mechanism

It is possible to cure sickle cell disease by a bone marrow transplantation. Unfortunately, most patients do not have a donor for this treatment. In addition, a bone marrow transplant is a risky treatment. Our new treatment first collects a sickle cell person's own blood stem cells and uses a new technology called CRISPR to correct the sickle gene in the blood stem cells. These are returned to the same person after first destroying the sickle-producing blood cells. It might stop the disease.

Unmet Medical Need

Currently, there are only two approved treatments for sickle cell disease, which are drugs that help treat symptoms but do not cure the disorder. There is an unmet need to approve new treatments that eliminate the cause of the disorder that arises in the blood cells, with potential of cure.

Project Objective

Obtain an IND an early phase clinical trial

Major Proposed Activities

- Find all the sites in human DNA where the CRISPR changes the code and confirm these changes are not dangerous or cause cancer
- Find all the types of the hemoglobin protein that might be made after the CRISPR fixes the sickle gene and confirm the hemoglobin in red cells is safe
- make enough of the gene-corrected blood stem cells to treat 3 patients and show these are safe in mice and have a good shelf-life after freezing

Statement of Benefit to California:

Sickle cell disease, a hereditary blood disorder that primarily affects individuals of African descent, is estimated to affect more than 6000 persons in California. Most adults die of the disorder by their late 40s. A curative therapy given early in life would have a significant beneficial effect on lifespan and the quality of life, and reduce life-long healthcare costs to families and to society. The goal of this proposal is to offer better treatment for every person with the disorder.

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